JCR Pharmaceuticals Co., Ltd.

Q2 Financial Results Briefing for the Fiscal Year Ending March 2024

November 2, 2023

Event Summary

[Company Name]	JCR Pharmaceuticals Co., Ltd.
[Company ID]	4552-QCODE
[Event Language]	JPN
[Event Type]	Earnings Announcement
[Event Name]	Q2 Financial Results Briefing for the Fiscal Year Ending March 2024
[Fiscal Period]	FY2024 Q2
[Date]	November 2, 2023
[Number of Pages]	35
[Time]	13:00 – 14:31 (Total: 91 minutes, Presentation: 47 minutes, Q&A: 44 minutes)
[Venue]	Webcast
[Venue Size]	
[Participants]	
[Number of Speakers]	7

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Toru Ashida	Senior Vice President, Sales Executive Director, Sales Division
Mathias Schmidt, PD,Ph.D.	Vice President, Clinical Development (overall supervision) Business Development and IR Fields, excluding Japan
Hiroyuki Sonoda, Ph.D.	Vice President, Research Executive Director, Research Division
Yoh Ito	Senior Corporate Officer, Corporate Strategy Executive Director, Corporate Strategy Division
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		and Director, General Affairs Dept. and Director, Human Resources Planning Dept.
	Yoshihiro Ohta	Director, Accounting Department, Corporate Strategy Division
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	Shinichiro Muraoka	Morgan Stanley MUFG Securities
	Hiroyuki Matsubara	Nomura Securities
	Shinya Tsuzuki	Mizuho Securities
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*Analysts that SCRIPTS Asia was able to identify from the audio who spoke during Q&A or whose questions were read by moderator/company representatives.

Presentation

Moderator: JCR Pharmaceuticals FY2023 H1 financial results briefing is starting.

First, let me explain today's language settings. At the bottom of your Zoom window, there is an interpretation icon. Please select off, Japanese or English channel.

Before I begin, I would like to remind the audience that this presentation may contain forward-looking statements based on current expectations. In the following presentations, we may make forward-looking statements based on our current expectations, which are subject to risks and uncertainties. Please be aware that actual results may differ materially from those projected in the forward-looking statements.

In addition, today's presentation and the materials used today are intended to provide information about our business to shareholders, investors, and the media. Information regarding products under development and pharmaceuticals is not intended as advertising or medical advice nor is it intended as a guarantee of future results or the indication of products under development. Please note that this briefing is being recorded for posting on our website at a later date.

To begin, I would like to introduce the speakers. Representative Director, Chairman, President and CEO, Shin Ashida; Senior Vice President, Executive Director, Sales Division, Toru Ashida; Vice President, Mathias Schmidt; Vice President, Executive Director, Research Division Hiroyuki Sonoda; Senior Corporate Officer, Executive Director, Corporate Strategy Division, Yoh Ito; Senior Corporate Officer, Executive Director, Administration Division, Yutaka Honda; Director, Accounting Department, Corporate Strategy Division, Yoshihiro Ohta. These are the seven speakers.

I would like to explain the materials that we are using today. The materials were posted on our website at 5:00 PM. on November 1. If you would like to have the materials at hand, please refer to our website. Next, I would like to provide you with an overview of the flow of today's briefing. Today's presentation will last approximately one hour and 30 minutes, including the presentation and Q&A session. Questions will be taken after all presentations have been completed. The Q&A session will last approximately 50 minutes.

Now, the Chairman, Ashida, will give opening remarks. Chairman Ashida, please.

Shin Ashida: This is Ashida. To all, I would like to thank you for your deepest understanding and support to our company. At today's financial results briefing, first, by 2030, we would like to share how we would like to be as a company. After that, we will share the overview of FY2023 H1 financial results.

This fiscal year, GROWJECT, our core product, is steadily growing due to the supply issue of other companies. Also, IZCARGO and TEMCELL as well, we are seeing growth more than what we expected. Vaccine manufacturing contributed quite a lot two years ago. Except that, we are achieving the level close to all-time high in sales as well as profit.

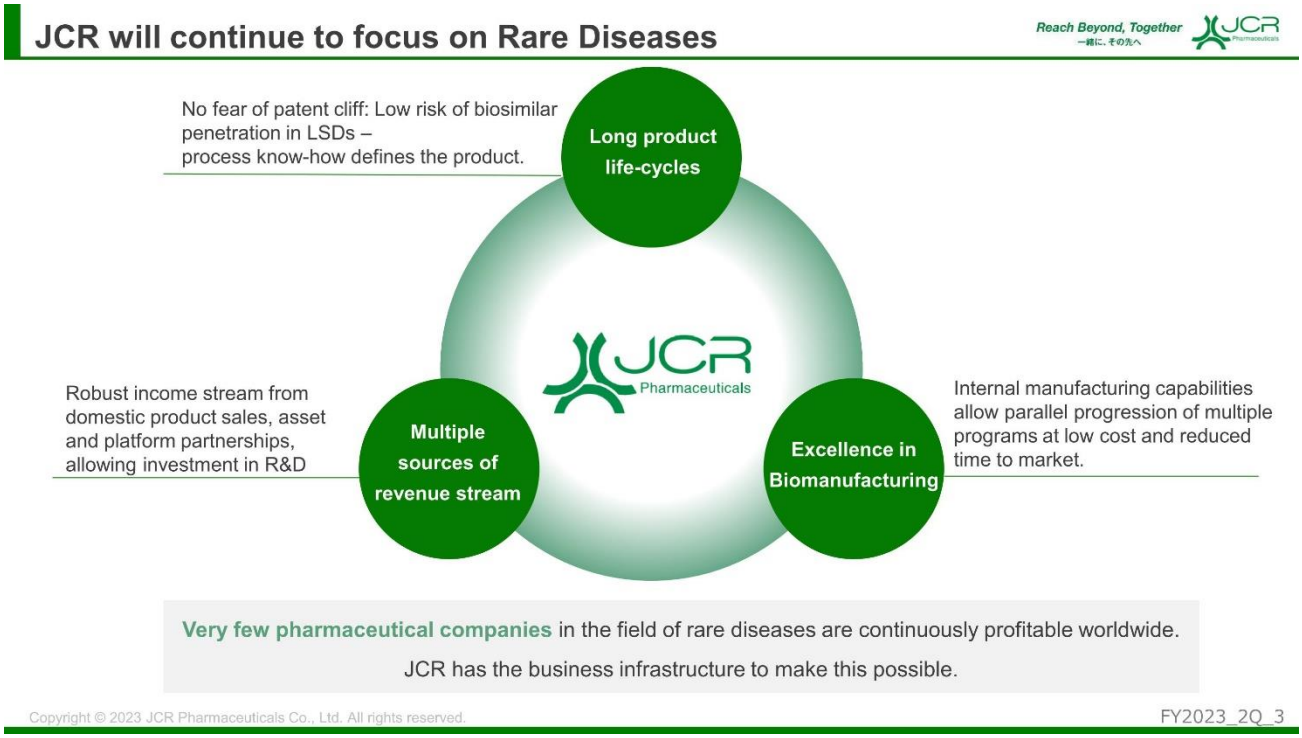
As a new product drug development, mainly with the LSD (Lysosomal Storage Disease) area using J-Brain Cargo technology, our first JR-141, IZCARGO, was launched. Following that, JR-171, clinical Phase I/II study results were gained. We had quite good clinical results. With this, the J-Brain Cargo is effective in other LSD enzymes, which was shown. Therefore, for our development pipeline in LSD, we expect it will go well.

As for JR-441, global Phase I/II study has seen the first patient in Germany administrated. Therefore, it is going well. Following that, JR-446, in sequence will enter into clinical stage. We would like to also look into the opportunities for licensing so that the drug can be administered in a wider range of patients, and we are

negotiating the licenses. The LSD area, as well as the licensing to other companies, as well as royalty, by 2030, we would like to achieve the sales close to JPY100 billion.

We will continue our contribution into rare disease. In the world, there are very few companies that are only working in rare diseases. However, JCR is determined to take on a challenge in rare diseases. That is what we would like to follow through. Moving forward, I would like to ask your support. Thank you very much.

Moderator: From here, we would like to present the financial results overview as well as our business and by respective responsible persons. From Ito, first, he will present "JCR's goal in mid 2030s."

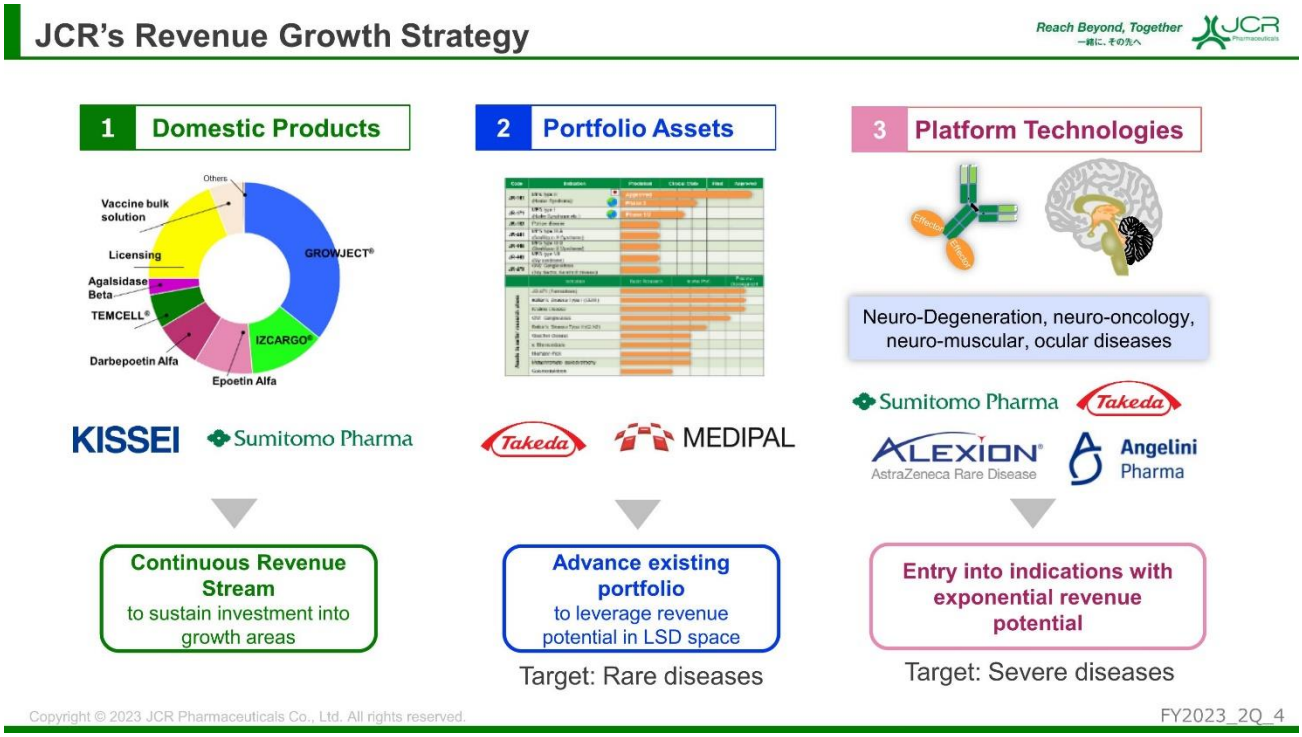


Ito: I am the head of the corporate strategy division, and I would like to present "JCR's goal." As Ashida mentioned, in the world, specializing in rare diseases and continuing to achieve profits, there are only very few companies there. We do have the business foundation enabling us to do that. There are three foundations.

One is we are focusing on the area of LSDs that has a low risk of biosimilars entering the market. The LSDs area, looking at individual diseases, there are very small markets. Therefore, there is a very low penetration of biosimilars. By producing the advanced products, there will be no fear of patent cliff in order to generate the profit. That is how I would like to generate our products.

The second foundation is our multiple sources of revenue stream. For example, rare disease area such as LSDs and the domestic product as well as J-Brain Cargo technology are being licensed out. We will be able to approach into so many different disease areas. With that, in the long term, we can continue to invest in R&D in rare diseases.

Third is biomanufacturing technology as well as the equipment that we have. By internally having that capability, multiple products can be simultaneously produced, and we can actually provide the products at low cost and reduce our time to the market. With that, we can continue to focus on the rare disease area as a pharmaceutical company.



Next, please. Let me talk about our growth strategy.

As I mentioned earlier, in Japan, we have a sales foundation. GROWJECT, IZCARGO, and TEMCELL are other representative products that we have. With the sales of our core products, we can actually generate stable profit. Secondly, we have the portfolio in LSD. By advancing in the LSD arena in terms of the development and partnering, we can secure the long-term revenue. Third is the platform technology. We have a J-Brain Cargo technology. With this, we can see significant revenue. We can actually enter into different areas, disease areas, to make the profit with a partnering strategy.

More Stable Profit Structure

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Enhanced sales from GROWJECT® enabled a more stable profit structure, allowing to cover R&D and SG&A expenses solely through revenue from domestic products.

Next, please. In terms of the profit structure, I would like to add.

From this year, we are able to have stable revenue foundation. As on the left, R&D as well as SG&A, we could not actually cover only the profits from the domestic products until last year. That means that we were relying on a licensing fee. However, from this year, with the growth of our domestic products revenue, a stable revenue structure, R&D and SG&A, we can actually cover those costs with only the profit made by the domestic products. Then, we can actually continue our stable foundation.

Estimated Timeline to Approval and Revenue from LSD Assets

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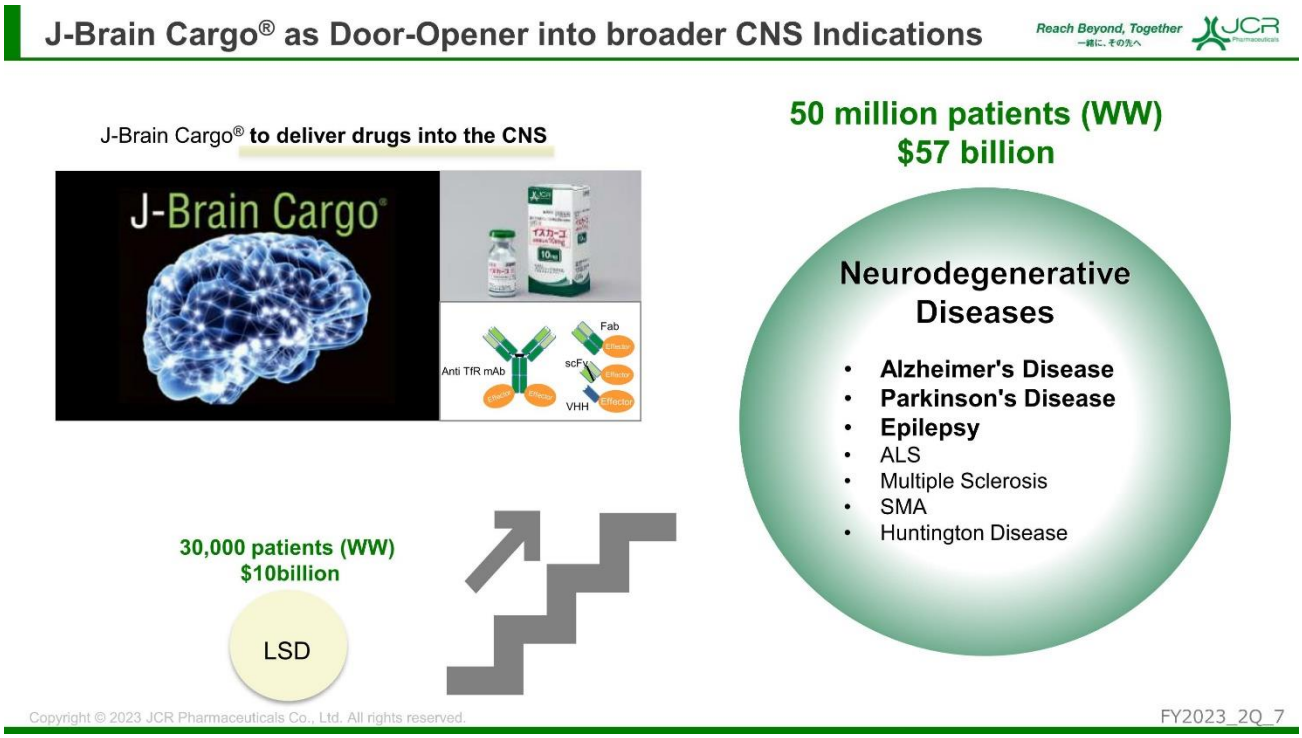
	Stage	Estimated time to approval	Revenue potential* (bn yen; by mid-2030s)		Global Partner
		FY2025 FY2030 FY2035	Japan	Ex-Japan	
JR-141 (MPS II)	P3 (Global) Launched (JP)	FY2027	8-10	14-18	Takeda
JR-171 (MPS I)	P1/2 (Global)	FY2028-2029	2-3	6-10	
JR-441 (MPS IIIA)	P1/2 (Global)	FY2029-2030	1-2	-10	
JR-446 (MPS IIIB)	IND enablement	FY2029-2030	1-2	-5	MEDIPAL
JR-479 (GM2)	Pre-clinical	FY2030-2031	2-3	10-15	
JR-471 (Fucosidosis)	Pre-clinical	FY2030-2031	<1	-5	MEDIPAL
		Total	15-20	50-60	

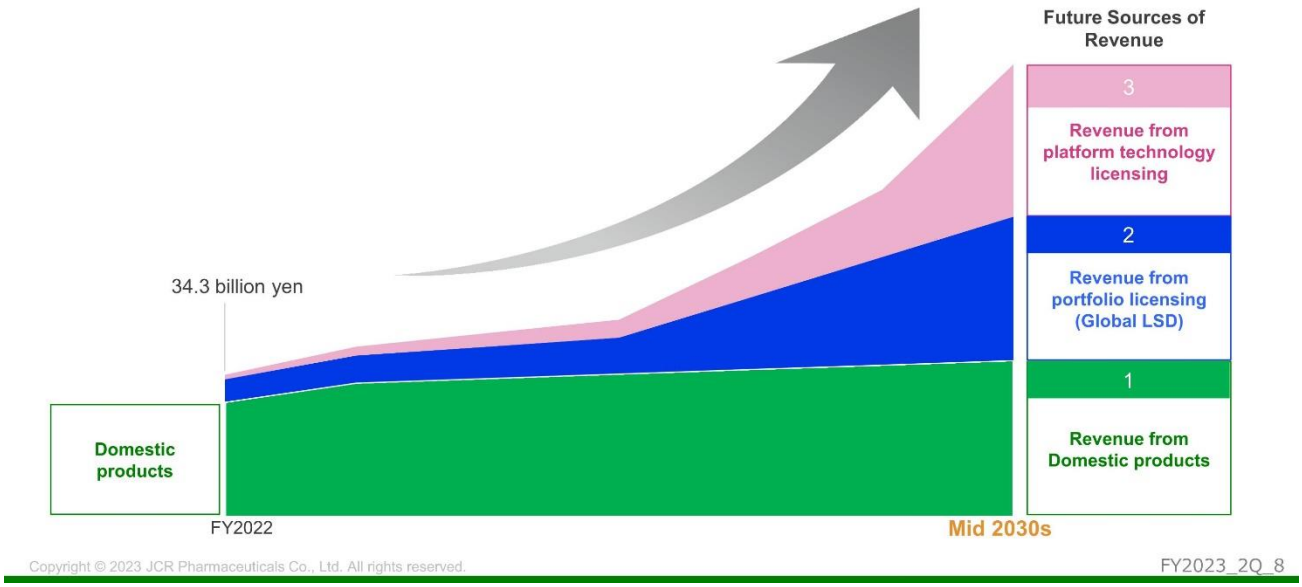
* Calculated by JCR based on est. market

Now, in the LSD market. Here, it shows our ideal situation in the future.

On the left-hand side, we have JR-141, JR-171, JR-441, and JR-446. These are the products that we have. These are the portfolios we have. What are the possible pipeline timetables? As you can see on the right-hand side, in the middle of 2030, JPY15 billion to JPY20 billion in the domestic market. Overseas, this is royalty revenue through the partnering. We expect JPY50 billion to JPY60 billion worth of the revenue from that kind of alliance overseas.



Next slide, please. Here shows other brands than the LSD market, our own J-Brain Cargo, the technology. It can bring us the opportunity to move into the CNS (Central Nervous System) areas. Looking at LSD, we have about USD10 billion worth of the market. However, we can see about USD57 billion at CNS, the market which can be explored through this technology, J-Brain Cargo technology.



Next slide, please.

So far, I talked about three revenue pillars. One is the sales coming from the core domestic products. The second is a license in the area of LSD. The third is J-Brain Cargo technology license revenue that is coming from the overseas market. By having the established revenue, plus in the middle of 2030, we can have revenue amounting to JPY100 billion.

The second pillar, LSD pipeline revenue, and also the third pillar, the platform technology revenue, basically, through the partnering, we will be able to generate the revenues from the overseas market. Those two have to do with the royalty revenue. In other words, it does not have any cost. In other words, we can enjoy the high level of the revenue. This is the explanation of JCR Pharmaceuticals for the future.

I would like to ask now, Ohta to come up to the stage to explain the consolidated financial results.

Ohta: This is Ohta from accounting department. 2024, the fiscal year, I would like to explain to you the H1 financial results.

Financial Highlights（Apr. 1, 2023- Sep. 30, 2023）

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Strong sales of core products and higher contract income contributed to a significant year-on-year increase in revenues and profits.

- Net sales : 24,272million yen （YoY +55.8%）
- Operating income : 6,898million yen （YoY +826.2%）
- Ordinary income : 7,126million yen （YoY +353.9%）
- Profit attributable to owners of the parent : 5,253million yen （YoY +445.6%）

First page, please. You can see the financial highlights.

I may repeat the same thing. Core product sales were quite good. Also, license revenue has increased. 55.8% plus against the previous average is JPY24,272 million, a huge amount of sales we were able to generate. With it, we will be able to see good results in terms of income. Operating income, JPY6,898 million, ordinary income JPY7,126 million. Profit attributable to the owners of the parent, JPY5,253 million.

Breakdown of Net Sales

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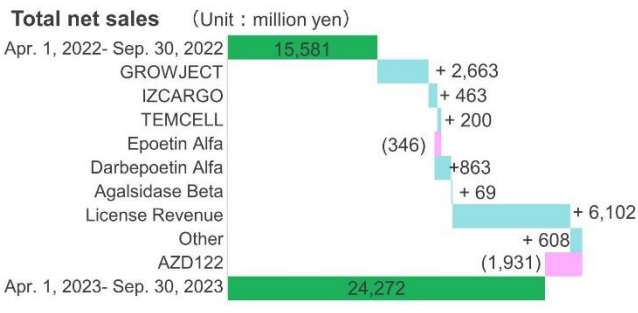
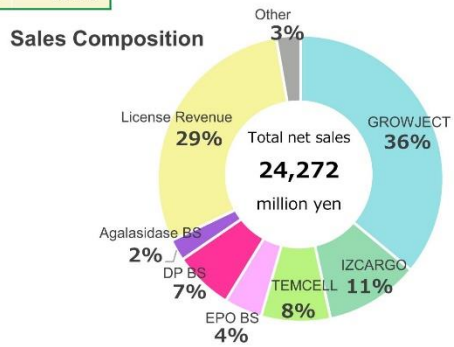
(Unit: million yen)

	FY2022	FY2023				
	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023	YoY Difference	YoY Ratio	Full year Forecast (Revised)	Progress Rate
Core products	12,583	16,495	+3,913	+31.1%	34,700	47.5%
License Revenue	1,010	7,112	+6,102	+604.2%	8,100	87.8%
Other	56	664	+608	+1085.7%	2,600	25.5%
AZD1222 bulk	1,931	—	(1,931)	—	—	—
Total net sales	15,581	24,272	+8,691	+55.8%	45,400	53.5%

- Core products sales increased 31.1% YoY due to strong sales of GROWJECT® and other core products, IZCARGO® and TEMCELL®.
- License revenue and other sales (including contract manufacturing) progressed as planned, resulting in a YoY increase.





Next slide, please. This just shows a breakdown of net sales.

Starting from GROWJECT, we have IZCARGO and TEMCELL. We have all these core products. Core products are moving along very nicely. The pharmaceutical sales against the previous year, 31.1% plus, JPY16,495 million. License revenue, JPY7,112 million, and others, JPY664 million. All the fees are according to the plan. With that, JPY24,272 million we enjoyed as a net sales.

Operating Income

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(Unit: million yen)

Consolidated	FY2022 Six months ended Sep. 30, 2022	FY2023 Six months ended Sep. 30, 2023	YoY Difference	YoY Rate	Full year Forecast (Revised)	Progress Rate
Net Sales	15,581	24,272	+8,691	+55.8%	45,400	53.5%
Cost of sales	4,836	5,881	+1,044	+21.6%	12,400	47.4%
Gross Profit	10,744	18,391	+7,646	+71.2%	33,000	55.7%
SG&A	5,782	5,957	+174	+3.0%	12,800	46.5%
R&D	4,216	5,535	+1,318	+31.3%	9,700	57.1%
Operating income	744	6,898	+6,153	+826.2%	10,500	65.7%

	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023
Ratio of cost of sales	31.0%	24.2%
Ratio of cost of SG&A	37.1%	24.5%
Ratio of cost of R&D	27.1%	22.8%
Operating income ratio	4.8%	28.4%

Operating income (Unit : million yen)

- With the growth in net sales, operating income increased significantly by 826.2% YoY.
- As a result of active R&D activities, R&D expenses increased 31.3% to 5,535 million yen (up 1,318 million yen YoY).

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Next page, please. This is operating income.

As I have mentioned, the net sales, we see the big numbers here, increased numbers. Along with it, gross profit, JPY18,391 million, which is plus 71.2%. SG&A against the previous year, plus 3%, which is JPY5,957 million, and aggressive R&D activities are in place. The R&D cost was plus 31.3% increase, which is JPY5,535 million. With this, the operating income was against the previous year plus 826.2%, JPY6,898 million.

That's the summary of the financial results. Thank you very much for your kind attention.

Moderator: Next, Senior Vice President, Toru Ashida is going to explain "JCR activities for further growth, domestic sales products."

Toru Ashida: This is Ashida speaking, from sales.

Sales of Core Products

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Strong sales of core products contributed significantly to the large increase in profit and sales.

	FY2022		FY2023				
	Full Year	Six months ended Sep. 30, 2022	Six months ended Sep. 30, 2023	YoY Difference	YoY Rate	Full year Forecast (Revised)	Progress Rate
GROWJECT®	12,261	6,083	8,746	+2,663	+43.8%	19,500	44.9%
IZCARGO®	4,428	2,118	2,581	+463	+21.9%	5,500	46.9%
TEMCELL®HS Inj.	3,404	1,701	1,901	+200	+11.8%	3,300	57.6%
Treatments for renal anemia	4,696	2,157	2,674	+517	+24.0%	5,000	53.5%
Epoetin Alfa BS Inj. [JCR]	2,710	1,392	1,046	(346)	(24.9%)	2,200	47.5%
Darbepoetin Alfa BS Inj. [JCR]	1,986	765	1,628	+863	+112.8%	2,800	58.1%
Agalsidase Beta BS I.V. Infusion [JCR]	964	521	590	+69	+13.2%	1,400	42.1%
Total Core products	25,755	12,583	16,495	+3,913	+31.1%	34,700	47.5%

I would like to talk about the domestic sales products. Next slide, please.

First, this shows the domestic sales results. As was mentioned, the main core products moving along are quite good and steady in terms of sales and profit. The top three products, which are promoted by our company, GROWJECT, IZCARGO, and TEMCELL, we see other bigger contributions. For these three products starting from this year, we have the specialist structure for the sales force. We have specialized and intensive promotions for each product.

GH Market Trends in Japan

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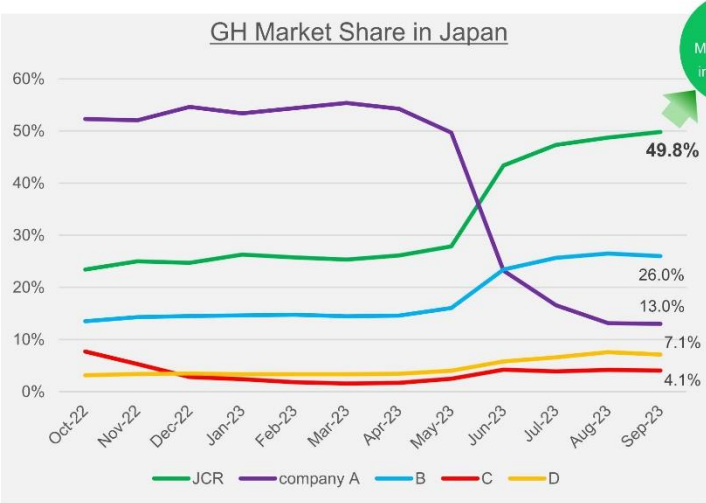
Demand for GROWJECT® has surged since May 2023.
JCR successfully achieved to increase production and stable supply of drug to patients.



FY2023 1st-Half Results*

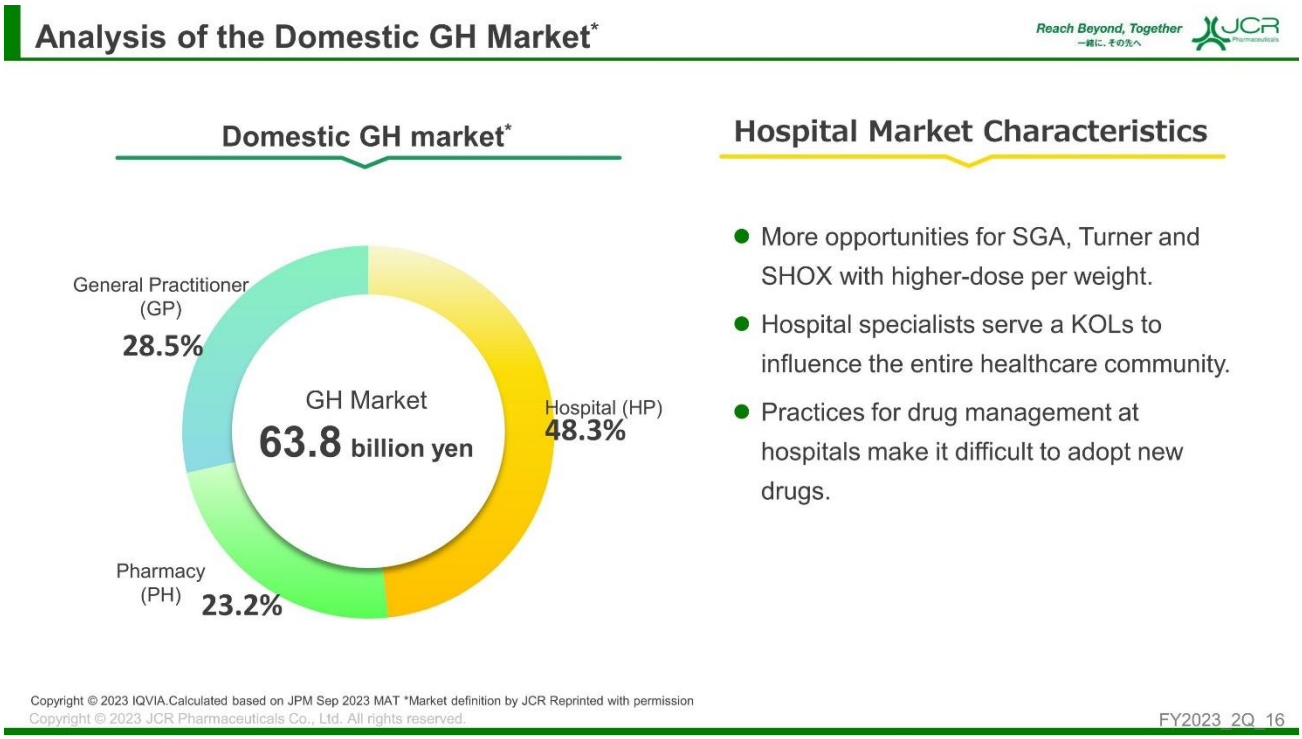
- Number of units shipped YoY approx. 1.6x
- Number of Naïve patients YoY approx. 1.8x
- Number of switched patients YoY 90 times more

*JCR internal analysis

Next slide, please. Now, let's about the growth hormone market trends.

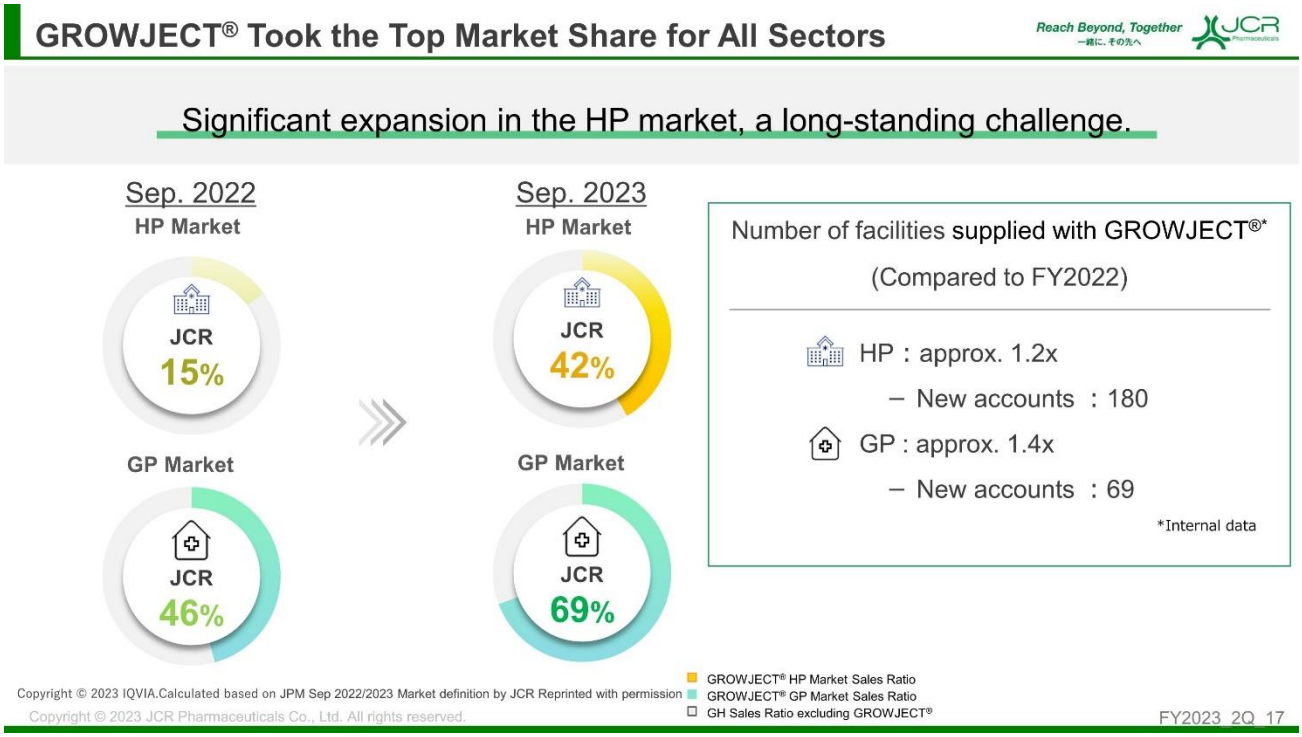
In 2023, May, there was a supply issue of other company's GH(Growth Hormone). With that, the demand for GROWJECT increased a great deal. We are the only Japanese company producing GH. To respond to this other demand, we increased products as well as devices. With this initiative, a stable supply of both product and device were in place. As a result of our efforts, as you can see in the graph, this shows an initial price base monthly results. About 50% market share was obtained in the recent months.

Also, on the right-hand side, you can see the H1 result of GROWJECT. The number of units shipped against the previous year, 1.6 times as much; number of Naïve patients, 1.8 times as much of last year; and a number of switched patients 90 times or more. Huge increase was achieved.



Next slide, please. It shows the domestic GH market.

On the left-hand side, you can see the pie chart, GH market in Japan, NHI price based market, about JPY64 billion market. Out of that, the hospital and the pharmacy, basically 60% having to do with the hospital market and the remaining 30% is coming from GP, general practitioners. So far, it's been a challenge for us to explore the hospital market. There are so many specialists in hospitals, SGA, Turner, SHOX, basically, those patients are found in HP. KOLs in hospitals have an influence on local healthcare. Then, when it comes to the adaptation, there is one drug per therapeutic area, which is the rule in place in hospitals, so it's very difficult to approach those HPs.



Next slide, please.

Given that background, in H1, with the growth of demand of GROWJECT, our challenge in growing in the hospital area, we were able to achieve that. As you see on the left-hand side, as of September 2022, this is the share by market. GP market, we had about 50% as of September 2022. In H1, as of now, we were able to grow our share in both the hospital market and GP market quite significantly. On the right-hand side, specifically in the hospital market, the new accounts were 1.2 times. Specifically, 180 new accounts were achieved. For a GP market, 1.4 times and 69 new accounts were opened.

Our Sales Strategy for Further Growth

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Strong and stable supply

- Already implemented a system to secure the production volumes necessary to maintain a market share of more than 50%
- Continued stable supply as GH top share manufacturer and increased market confidence

Device strategy

Two types of devices to meet the needs of patients and healthcare professionals

Growjector® L

- Full support for injections
- Enhanced compliance through fun features

Growjector® Duo

- Easier operation mode reduces reluctance to switching devices from other devices
- Reduced injection teaching time at the time of prescribing

Entering more new patients and sustaining existing patients

Next, please.

In order to further grow our share, first, we need to achieve a stable supply. Also, we need to have our device strategy in order to grow our shares, especially to maintain our top share. We were able to secure the production volume necessary to maintain that. As a top share manufacturer, I would like to also have increased confidence from the market. As for the device strategy, as a GH manufacturer in Japan, we provide the motorized injector. We are the only manufacturer to do that, and I would like to speak about that.

Motorized Digital Injector “GROWJECTOR®”

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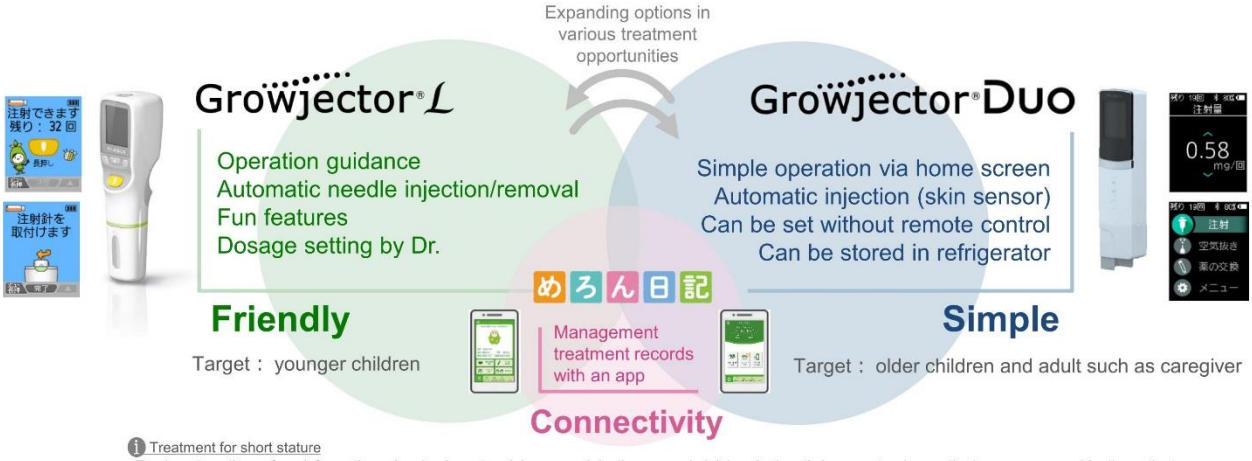
Providing motorized digital injectors that suit the patient's needs/lifestyle

Main features of motorized digital injector：
Constant speed of drug infusion, pre-setting and automatic calculations of doses, automatic adjustment of doses during drug changes, recording of injection history, etc.

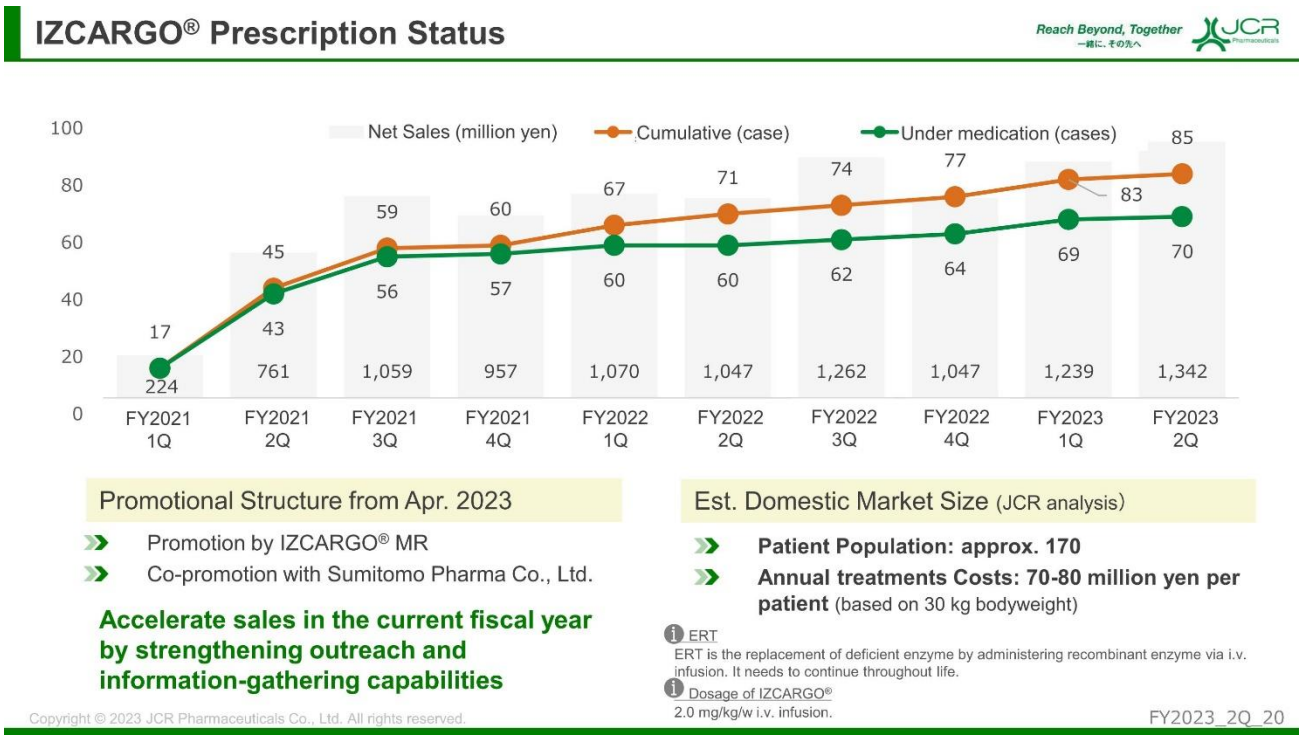


Treatment for short stature
Treatment continues from infancy through school age to adolescence. Injections are administered primarily by parents when patients are young, and by the patients themselves as they get older.

Next slide, please.

This is our motorized digital injector, GROWJECTOR. As you know, the GH treatment actually asks for the injection at home. Therefore, patients, as well as the family themselves, are self-injecting. We were trying to reduce the burden on that in our development effort with PHC Corporation. On the left-hand side is GROWJECTOR L, this is our main product. From this September, we also launched GROWJECTOR Duo. This is a new option added. The younger children is targeted with GROWJECTOR L. This can be used from quite a small age.

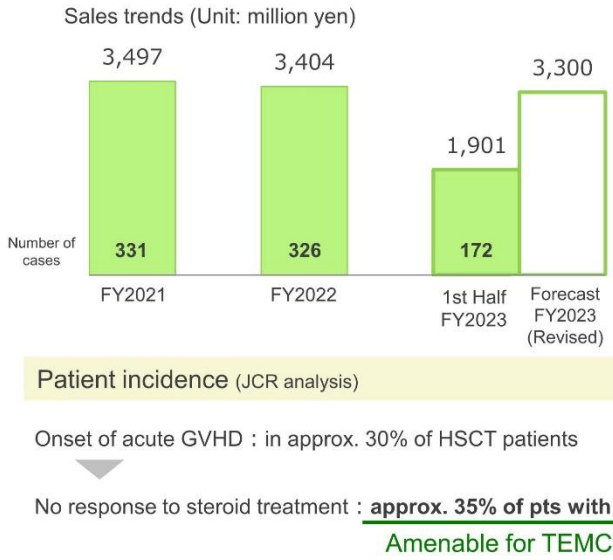
GROWJECTOR Duo, we made the operations more simple, older children, in the upper levels of the elementary schools, as well as the adults such as caregivers. With these two devices, we are trying to reduce the burden on them as well as on health care professionals. Furthermore, we believe that these two devices can reduce the burden not only the existing patients but also new patients. We would like to grow our share in H2 with these options.



Next, please. This is IZCARGO sales.

This is the cumulative cases and under medication cases, which are progressing as planned as well as on budget. As I mentioned earlier, from April this year, we have reorganized our sales structure, appointing specialized sales reps and also co-promotion with Sumitomo Pharma. We are enhancing our efforts in collecting information and would like to accelerate our sales in H2 with these efforts.

TEMCELL® Prescription Status



Patient incidence (JCR analysis)

Onset of acute GVHD：in approx. 30% of HSCT patients

No response to steroid treatment：**approx. 35% of pts with aGVHD**

Amenable for TEMCELL®

TEMCELL® sales status update

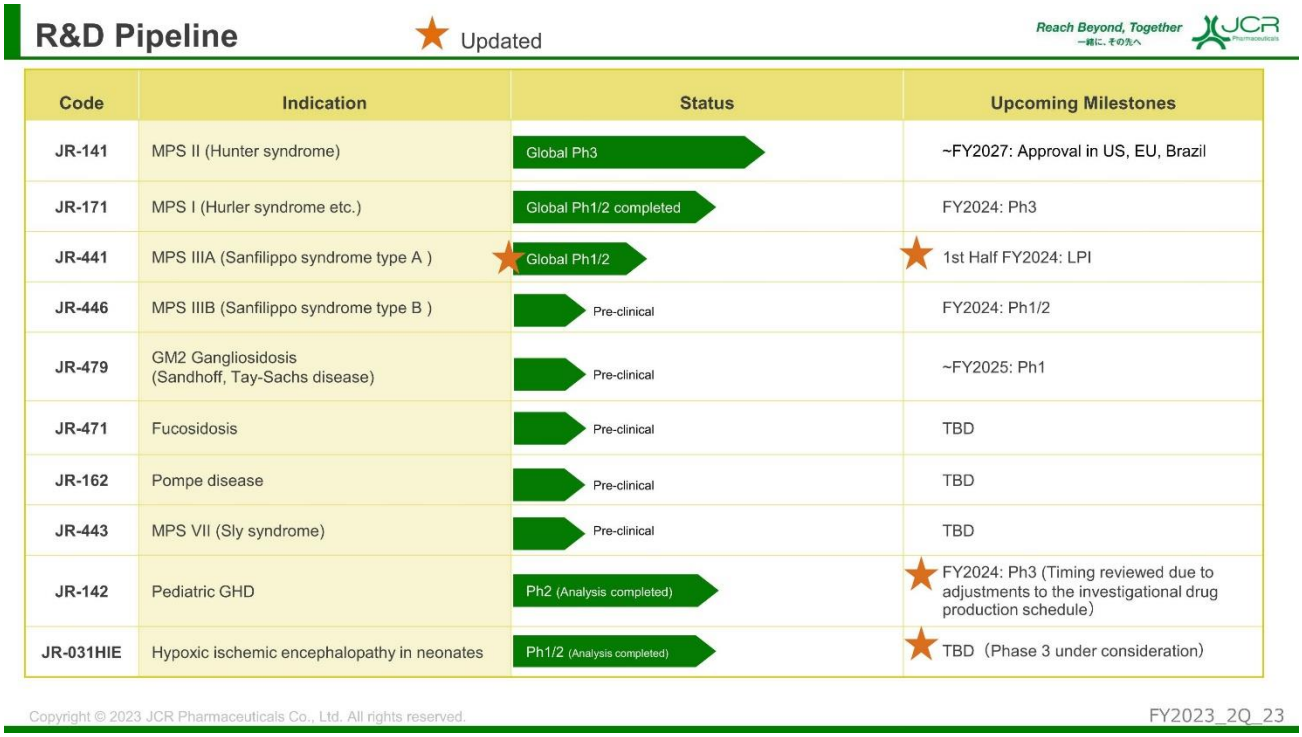
- **Available in more than 90% of target facilities**
- With the cooperation of MEDICEO CORPORATION, the lead time was reduced from 3 to 2 days.
- Changes in the prevention and treatment of aGVHD: Impact currently is not significant.
  - —24, Jul -2023：
    Cyclophosphamide hydrate is covered by insurance for the prevention of GVHD.
  - —23, Aug-2023：
    Ruxolitinib Phosphate has been approved for the treatment of GVHD after HSCT that is unresponsive to steroids.

Next, please. This is the TEMCELL sales.

As for TEMCELL, we are also growing steadily. Patients with an onset of acute GVHD, among them, no response to steroid treatment, approximately 35% of patients are for TEMCELL indication. We have a solid positioning in terms of the balance between safety and efficacy. Especially in Gastrointestinal symptoms with acute GVHD patients, we hear TEMCELL is becoming a first choice of drug. In 90% of the target facilities, we can deliver this drug. With the cooperation of MEDICEO CORPORATION, we were able to reduce the lead time by one day, which enables earlier administration in contributing to more patients.

With this reduction of lead time, we have gotten appreciation from doctors and KOLs as well. Also, in the prevention and treatment of acute GVHD, there are changes. However, there is a very limited impact to TEMCELL sales currently. This concludes my sales presentation on core products.

Moderator: Next, from Schmidt, we will have “JCR activities for further growth, R&D”, please.



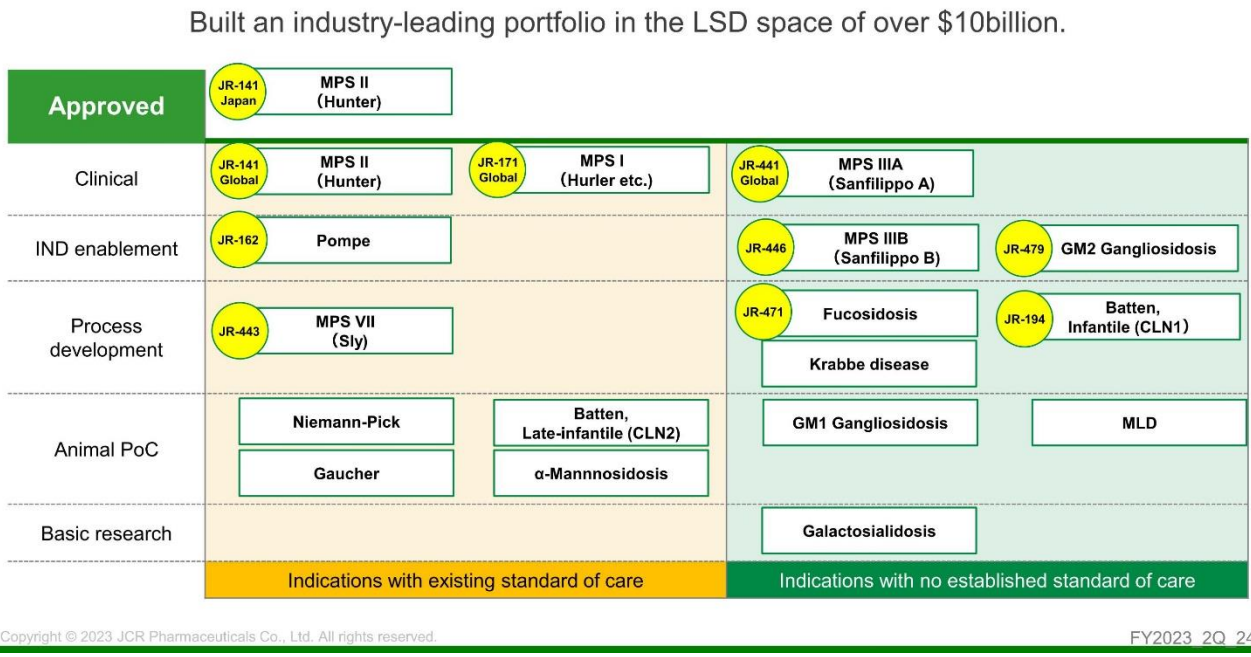
Schmidt*: If we can go to the next slide, please.

On this slide, you see an overview of our R&D pipeline of those assets that are either in the clinic or that are relatively close to the clinic. Of course, one of our most important programs is JR-141 in the global Phase III, together with our partner, Takeda Pharmaceuticals.

You might be aware, for our second asset, JR-171, we very recently published 52 weeks' data of our global Phase I/II study, which gave us very encouraging results and pointed out that the J-Brain Cargo technology does not only work for the treatment of MPS II but also for the treatment of MPS I. We are very pleased with the results. Something that we are also very proud of is the start of the JR-441 Phase I/II study in Germany, where we had the first patient dosed actually this month. In subsequent slides, I will tell you a little bit more detail about our MPS III assets.

You might also be aware about the partnering of our MPS IIIB assets, JR-446, together with MEDIPAL, and I will add some details about this molecule also in subsequent slides. Also, worth mentioning on this slide is our analysis of the Phase II JR-142, the long-acting growth hormone expected to further enhance our franchise in growth hormone deficiency. We will also show you some data from our Phase II analysis. Also, worth mentioning here is an indication expansion trial for our stem cells, JR-031, for the treatment of hypoxic ischemic encephalopathy in neonates, and we will also share some details in subsequent slides.

LSD Pipeline using J-Brain Cargo® Technology



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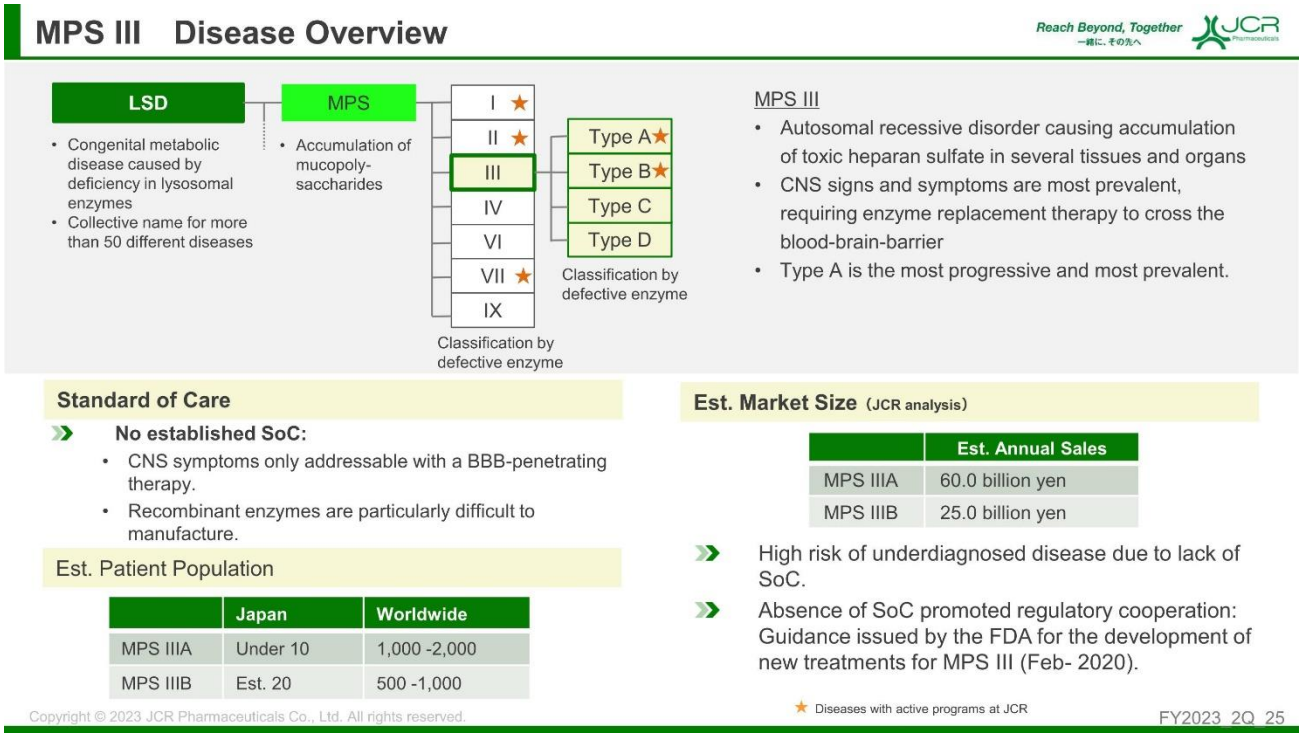
What you have seen on the previous slide is not our entire portfolio. Here, you see a comprehensive overview of our LSD pipeline built on the J-Brain Cargo platform technology. In applying this technology, we have built an industry-leading portfolio in the lysosomal storage disease space. If you see the combined market, it is actually more than USD10 billion involved.

You see on the left side that we have a full range of assets in indications where there is an existing standard of care, but we have a significant differentiation. We will bring game-changing innovation with our differentiated LSD assets that address the CNS disease burden in those diseases but also beyond, provide a much, much better delivery to other tissues and organs, including the muscle, spinal cord, and other organs as well.

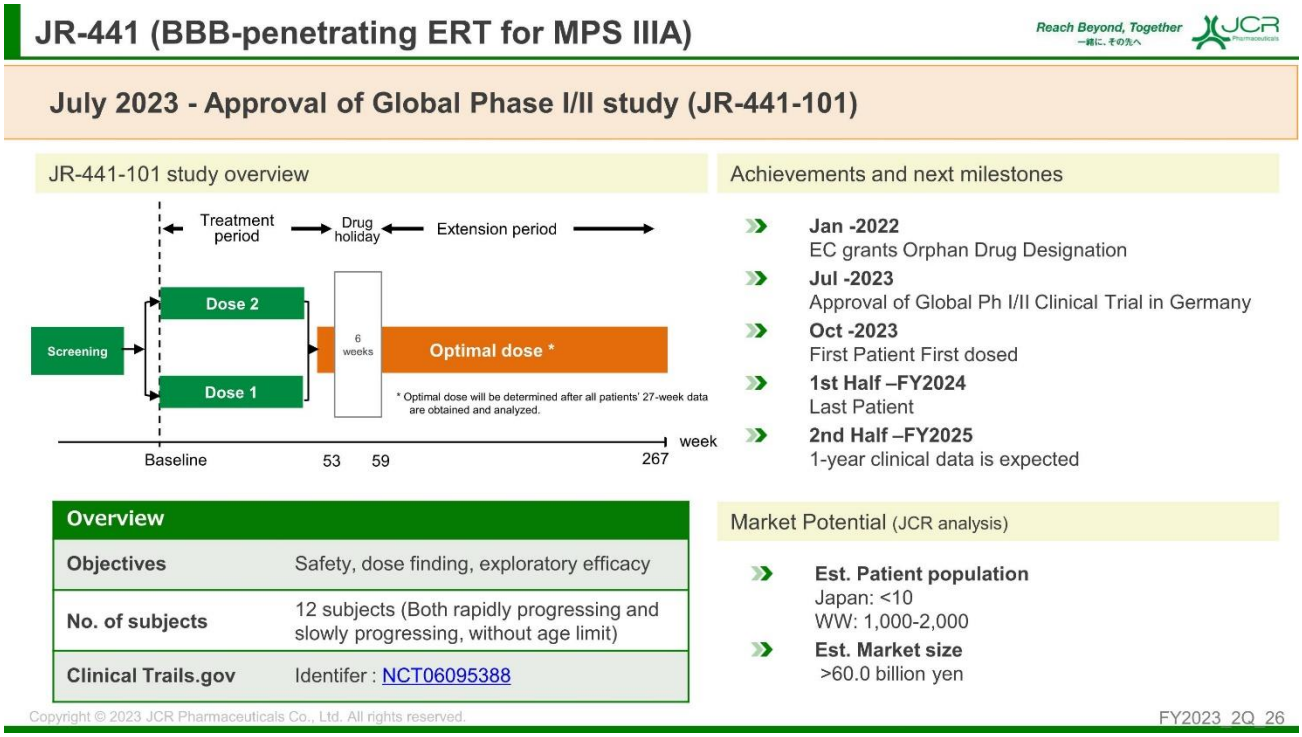
On the right side, you see assets that we have developed for the treatment of certain lysosomal storage diseases, where there is absolutely no standard of care established. We expect those assets to be real game changers for the treatment of these indications.



Next slide, please.

I would like to go a little bit into detail about the MPS III assets that we have in development. MPS III is a lysosomal storage disease. Mucopolysaccharidosis is actually a set of seven different indications. 7 to 11, if you consider the differentiation. And actually, MPS III, on Sanfilippo, is the largest set of mucopolysaccharidosis in the MPS space. Type A and Type B are the most prevalent forms. Type C and D are very, very rare forms.

What classifies MPS III or Sanfilippo syndrome is it is primarily a CNS-driven disease. In order to be successful, any drug developer must have a therapy that is able to cross the blood brain barrier. At the same time, these enzymes are challenging to manufacture. Any drug developer must have mastered the manufacturing of such therapeutics. I can say that JCR with J-Brain Cargo platform technology and its manufacturing capabilities has actually mastered those two major hurdles that prevent otherwise the entry in the MPS III space.



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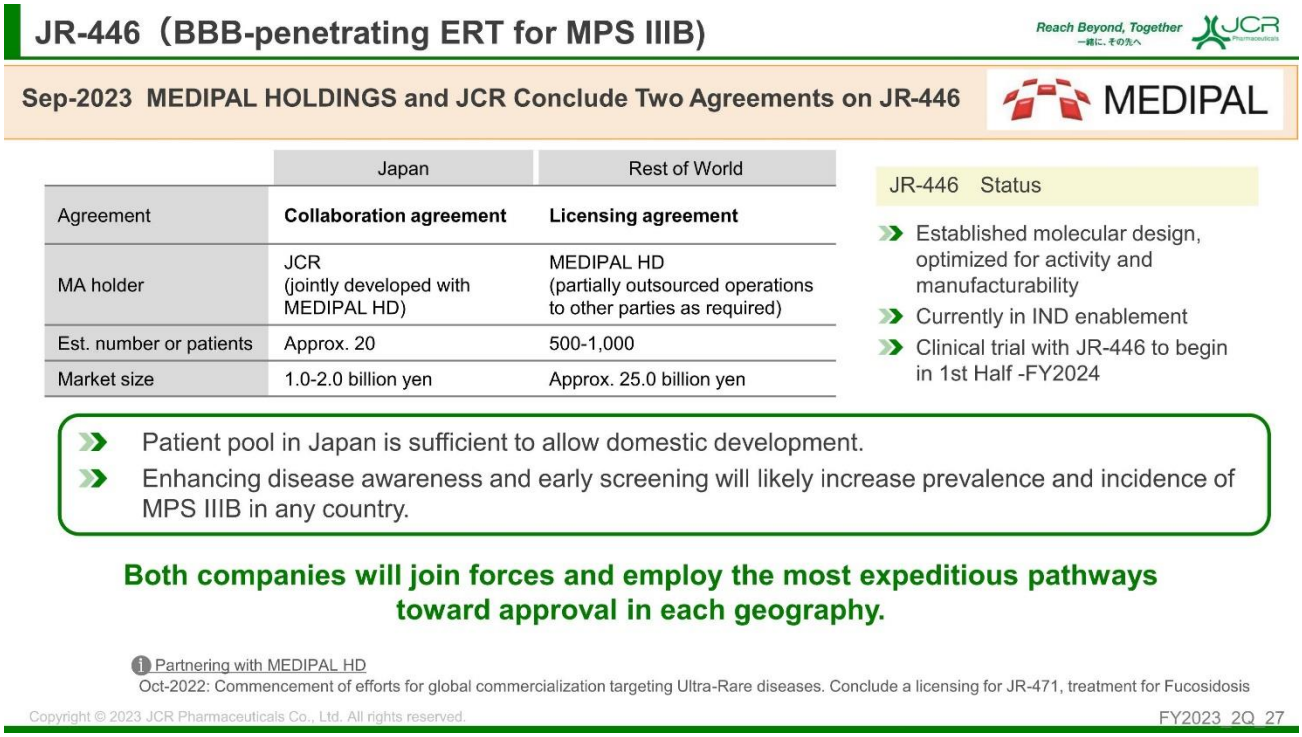
What you can see here is an overview of our global Phase I/II study that we have started, where we have dosed the first patients in October this year. We have designed this clinical trial together with expert clinicians in the field but also with parents of affected children with Sanfilippo A syndrome.

Our objective is not only to demonstrate the safety, the best dose, but we also want to explore the efficacy. We want to identify the best endpoints that are most sensitive to its drug interference and the patient population that is more sensitive towards drug interference so that we can design a pivotal study to come to approval with higher likelihood of success. This trial is already actually oversubscribed. As I said, we dosed the first patient this month, October 2023. We expect that we will enroll the last patient in H1 of FY2024 and have the one-year readout in H2 of FY2025.



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Another asset particularly addressing a disease with particularly high unmet medical need is JR-446 for the treatment of MPS IIIB. You can almost consider MPS IIIA and IIIB as sister indications. Whatever we will find out for MPS IIIA in terms of end points and sensitive patient population will likely be applicable also for the treatment of MPS IIIB. We are proud to have partnered with MEDIPAL for the development of JR-446. In Japan, we run this as a collaboration. For the rest of the world, we will run this as a license agreement where MEDIPAL has basically the license to market and commercialize the asset outside of Japan.

One big advantage in those ultra-rare diseases is that regulators are highly cooperative and lay out good regulatory pathways to come to approval as expeditiously as possible. Both companies will join forces to employ the most expeditious pathways toward approval in each geography.

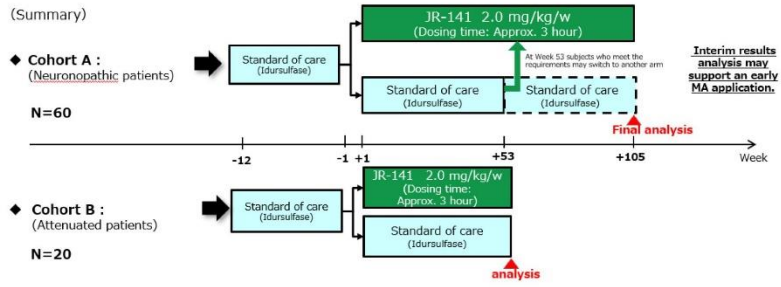
Here is another big advantage. JCR is present in Japan, in Europe, the United States, and Latin America. You can apply a tailored strategy to come to approval as expeditiously as possible for each of those regions. What I can say is the drug is currently in IND enablement, all our predicting data are extremely encouraging, and the molecule itself has been optimized for expression, for activity, and manufacturability. We are very pleased that this is a very well-behaved molecule, and we believe that we can start clinical trials in H1 of FY2024.

We believe there is a patient pool that is sufficiently big in Japan to allow domestic development, but we also believe that the availability of a drug and the implementation of early screening and raising disease awareness will most likely increase the prevalence and the incidence of the MPS IIIB and also the IIIA disease, probably in any country.

JR-141(pabinafusp alfa: BBB-penetrating ERT for MPS II)

Reach Beyond, Together 一緒に、その先へ JCR Pharmaceuticals

Global Phase III study（JR-141-GS31): STARLIGHT study Overview



Overview	
Objectives	1. To assess the efficacy of JR-141 on CNS signs and symptoms in MPS-II subjects relative to standard ERT 2. To assess control of somatic signs and symptoms by JR-141 relative to standard ERT
Endpoints	• Changes in HS in CSF, CNS symptoms (cognitive, behavior, attention) • Control of systemic sign and symptoms
Clinical Trails.gov	Identifier : NCT04573023

JCR Pharmaceuticals × Takeda

Upcoming milestone
1QFY2024 - all patients enrolled necessary for interim analysis

Current Status

- Recruiting
- Number of Clinical trial sites (as of Oct 2023):
  - USA: 5, Europe: 10, Brazil: 2
  - Further sites to open in EU, USA, LATAM and Asia to accelerate recruitment

Achievements

- **Oct -2018** ODD by FDA
- **Feb -2019** ODD by EMA
- **Feb -2021** Fast Track Designation by FDA
- **Oct -2021** PRIME Designation by EMA
- **Feb -2022** First Patient dosed in JR-141-GS31

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For JR-141, I can say enrollment is going well. We have 17 sites in the US, Europe, and Brazil active, and we are trying to activate more sites to further enhance and accelerate enrollment. We believe that in Q1 of FY2024, we will have all patients involved that are necessary for the interim analysis.

We are trying to achieve two objectives, and this is why we have two cohorts. They are trying to demonstrate that JR-141 has superior activity on the CNS signs and symptoms in MPS II compared to standard enzyme replacement therapy. At the same time, we would also like to demonstrate that the control of the somatic science and symptoms is as good as with standard somatic enzyme replacement therapy.

JR-142（Long-acting GH）

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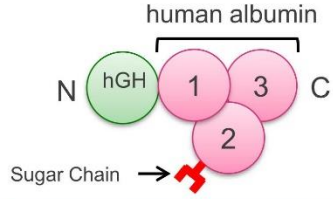
The growth promoting effect of JR-142 was confirmed in Phase II trial.
Phase III trial scheduled to start in FY2024.

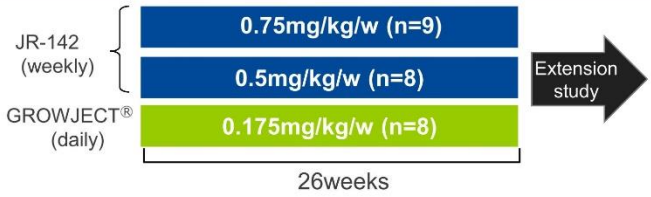
JR-142 molecular design

Modified albumin-fused GH



In-house development of fusion protein with modified albumin glycosylation to improve blood retention.

Phase 2 study design



Subjects	Pediatric growth hormone deficiency
Endpoints	• PK/PD • Changes in Height velocity • Safety
Details	jRCT(Identifier : jRCT2031200372)

Growth hormone treatment in Japan
Daily GH (5 products), Weekly GH (2 prodcuts)

Next slide, please.

I am moving away now from the LSD assets to long-acting growth hormone, which is a very significant part in our strategy to further enhance our growth hormone franchise in Japan. On the left side, you see the molecular design. It is a fusion protein with human albumin in order to increase the half-life in the circulation.

On the right side, you see the Phase II proof-of-concept study design, where we compared two different doses, 0.75 and 0.5 milligram per kilogram per week with daily doses of GROWJECT. We have included pediatric patients with growth hormone deficiencies, and we were looking for a growth velocity, the PK and PD profile and, of course, the safety.

JR-142 Ph2 results: Comparable Safety and Efficacy Profile

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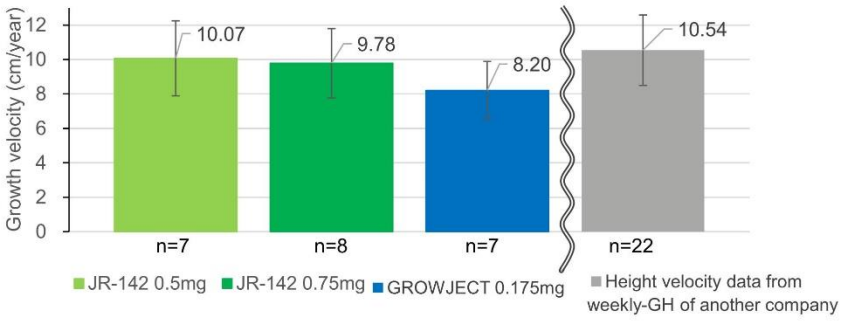
Key outcomes:

- Height velocity: Comparable between GROWJECT® and JR-142.
- AEs/safety profile: Comparable between all groups.

Outcome of Phase 2 Trial (height velocities)



Next milestones

- 2nd-Half FY2024 Start Ph3
- FY2027 File for Marketing authorization

Domestic Market Size

- approx. 18% of GH market share (as of Sep. 2023; JCR analysis)

Reference: List of growth rates after GH treatment (drawn in-house from internal data, published materials, etc.)

Next slide.

We were actually very pleased with the outcome. What we see is in terms of growth velocity, we have a comparable profile between both doses of projects and JR-142. When it comes to the adverse events and the safety profile, what we can say, it is also comparable between both groups. They have also included the growth velocity data from another company in order to demonstrate that the growth velocity with long-acting growth hormone JR-142 is very much comparable with the activity of other molecules that have long-acting activity.

We think that we can start our Phase III study in H2 of FY2024 and file for marketing authorization approximately in FY2027. We believe that there is approximately a slightly less than 20% market potential for long-acting growth hormone.

Other Activities to enhance Access to Medicine

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- JR-031HIE: Expanded indication of TEMCELL® HS inj. for Hypoxic ischemic encephalopathy in neonates
  (detail: jRCT1080224818)
  - Future development plan under consideration based on results of Ph I/II and 18-month observational studies.
    - Comparative study of hypothermia alone (7 subjects) and TEMCELL® plus hypothermia (6 subjects)
    - Over 65% of subjects in both groups were found to benefit from treatment after 18 months of treatment. However, no differences between groups were observed.
    - No safety issues were identified.

- Cooperation in investigator-initiated clinical trials
  - Provided investigational drug for "Phase II/III open study to evaluate the efficacy and safety of chaperone therapy with ambroxol hydrochloride (JT408T) in patients with neuronopathic Gaucher disease"

Next slide, please.

Two activities that I would like to briefly mention that JCR has conducted to enhance access to medicine is number one, a study that applies TEMCELL for the treatment of hypoxic ischemic encephalopathy in neonates. This is an indication with very high unmet medical need. We have done the clinical study. The clinical study is completed, and we did observe a very good activity and over 65% response rate in both treatment arms. There were no safety issues identified upon the treatment with TEMCELL.

The other activity to enhance access to medicine is we were supporting an investigator-initiated clinical study. It was a Phase II open-label study to evaluate the efficacy and the safety of a drug called ambroxol, which is approved in other countries as an expectorant but is considered to be a chaperone therapy for the treatment of patients with neuronopathic Gaucher disease. We are in the stages of analyzing the data as we basically speak.

I think this concludes my presentation. Thank you so much.

Question & Answer

Moderator [M]: Thank you very much. From here, we would like to move on to the question-and-answer session. How to ask a question is shown on the screen. When it is your turn, I will call your name. Please unmute your microphone and state your company as well as your name before asking questions. Please note that each person may ask up to two questions at a time in a question-and-answer format, but you may raise your hand as many times as you would like. Then, we would like to begin our Q&A session.

First, Yamaguchi-san, please.

Yamaguchi [Q]: This is Yamaguchi from Citigroup. One question regarding growth hormone. As explained, currently, the share is surely growing, and that is really great. On the other hand, the market itself is shrinking. I think that is a fact. I would like to ask your take on that as a company. Once the market comes back and then the share goes up, do you have inventory sufficient to supply? Rather than the share, looking at the market shrinking and also the supply capability are the questions that I would like to ask.

Shin Ashida[M]: Well, thank you for your question. I would like to ask Senior Vice President, Ashida, to take this question.

Toru Ashida [A]: Yes, let me respond to this question. Thank you for the question. As for the share, looking at the market trends, the difference in NHI pricing with other companies' products, the market, based on NHI pricing, is shrinking for sure. Also, the at-home inventory as well as the in-hospital inventory still exist. These are the two factors that we believe are there. In terms of the supply capability, all the companies are resuming their supplies. Even if we grow our share in that environment, we have sufficient supply capability already in place.

Yamaguchi [Q]: Briefly, regarding GROWJECTOR Duo, how was it received?

Toru Ashida [A]: As I mentioned earlier, there are no restrictions, but our concept of GROWJECTOR is from those patients getting treatment from infancy and growing older. There are apps with fun features, but it might be too childish. We wanted to provide a more stylish app. As I do ride along and go onsite, there are target patients as well as the health care professionals, not only them. I do feel that, overall, we do have a really great take on this. There is really great news that the patients who are switching to GROWJECT were able to inject themselves. That is what I heard.

Yamaguchi [Q]: Also, the second question, in terms of the licensing, the contents and also the timing won't be disclosed, and that's fine. However, in terms of the result up to Q2, there's good progress. Mainly, I believe that's the licensing fee from MEDIPAL might be licensing out. Regarding JR-171, there's additional data announced. Are you expecting to license out this year? What would be the activities for licensing out moving forward after next year? I'm sure that it is difficult to share the progress, but you can share the estimate.

Shin Ashida [M]: Ito will take this question.

Ito [A]: Well, thank you for your question. As for JR-171, we are working hard to negotiate with the companies for licensing out, and we would like to actually announce good news as soon as possible. Those are the activities that we are engaged in for JR-171.

Moderator [M]: Next question, from Muraoka-san, please.

Muraoka [Q]: Now, talking about the license fee. In terms of our budget, if you achieve another JPY1 billion, you'll be able to achieve the target. Remaining JPY1 billion, JPY1 billion is not really so big. Maybe the general progress will make it happen. Another six months, it may not be so much fun. It's sort of boring. You'll be able to achieve the target anyway. What is the possibility that some projects will move forward beyond the current budget, and within six months? Would there be any such fun news or not?

Shin Ashida [M]: Maybe Ito is going to respond to that.

Ito [A]: Thank you very much for your question. Yes, remaining JPY1 billion to go. At the previous meeting, we explained it. When it comes to the license fee revenue, we have a conservative view. Remaining JPY1 billion, we share that. Then for that, we are going to make steady progress to make it happen. When it comes to other projects with a bigger license scheme, this is to do with the previous question. Again, the negotiations are in place, and depending on how that negotiation with the outcome, the situation may be changed. When the time is right for us to make such announcement, we will do so.

Muraoka [Q]: You will be able to have the additional sum for this remaining six months. Am I right to say that?

Ito [A]: I don't know whether that amount is going to be a substantial amount or not, but let me tell you that we are doing everything possible. As quickly as possible, we would like to have the issue announcement to you.

Muraoka [Q]: Another question, competitive situation in the LSD. Orchard Therapeutics researching ex vivo gene therapy was purchased by Kyowa Kirin. Actually, the field is similar. In MPS Phase I/II, they will be into the pivotal test, and lead out is going to take place. I just found out that there is some kind of a competitor for JCR. This time, ex vivo genetic therapy and ERT, would you please tell us what are the positions? What are the commercial potentials? What are the possible strategies you may have against them?

Shin Ashida[M]: The comparison against Orchard, maybe I can ask Sonoda-san to respond.

Sonoda [A]: Thank you very much for your question. Orchard, yes, since before being purchased by Kirin, we have made a close look at them. That is ex vivo gene therapy, generally autologous transplantation. In other words, the cell is going to be extracted from the patient, a doctor modifies that cell, and then bring it back to the patient. It's such a cumbersome process. It is not an easy way to do treatment-wise.

It is not expanding because of this cumbersome and complicated process involved. When I discussed with the experts, one treatment covering all of that, that will not be the case. Actually, many doctors are looking for options depending on the conditions, gene therapy, maybe other options, and looking at the options is good.

What is easiest to do or use is ERT because it is ready to go, and you will be able to use it right away, a high level of safety. Safety is already proven in various types of rare diseases. ERT is the first choice. After, depending on the condition of the patients, probably genetic therapy can be chosen. In that sense, they are the competitor probably. Our Policy, focusing on the development for ERT that reaches the entire body including CNS symptoms, will never be changed.

Muraoka [Q]: I believe that gene therapy was in your scope as well and Orchard. As a purchaser, have you been interested? Regarding this, have you had an interest?

Shin Ashida [A]: Regarding Orchard, so far, we never thought about acquiring that company. As Sonoda mentioned, this is quite cumbersome in terms of processing the product. Universally, manufacturing the product is quite difficult. Therefore, we never thought about acquiring this company so far.

Moderator [M]: Next, Sakai-san, please.

Sakai [Q]: This is Sakai from UBS. Well, it might be a naive question. On page eight, becoming a company of JPY100 billion in 2030. I believe that you thought about this when you got feedback from the midterm business plan briefing. Regarding the three platforms or pillars, the domestic product sales, LSD pipeline, licensing out and platform technology, licensing all, can it clearly grow like these more? Ultimately, JPY100 billion is the number as an indicator, and you're just roughly dividing these three into that bucket.

Aligned with that, number three, the licensing income with licensing out platform technology, the J-Brain Cargo technology, when are you licensing out? The companies are interested in, and they have to have compounds or projects that can be added on to J-Brain Cargo. Otherwise, it will be a waste. The technology will be a waste. Therefore, in that sense, are you actively, I'm sure that looking into partners, do you have already multiple candidates or companies or projects? I believe Angelini or Alexion, I believe maybe both, whether there are any topics that come out moving forward.

Shin Ashida[M]: Regarding this, first, Ito will take the question.

Ito [A]: Well, thank you for your question. On page eight, there are three clearly divided pillars. As mentioned, we are showing that we will become a JPY100 billion company. These are quite rough. As a concept, the domestic product sales will steadily grow. As for the LSD, on the previous page, the timelines are shown. We believe that we can generate revenue from that's.

Catching up to that, number three, we'll generate, and number three will take the most time in our opinion. Regarding your next question, with the licensing income, with licensing out platform technology, regarding this already with Angelini and Alexion, we have concluded our agreement for licensing out our technology. Like this, we'd like to move forward as well. We are working on licensing out activities. Currently, we are in discussion with multiple companies. Once we conclude agreements, we would like to announce that as an outcome.

Sakai [Q]: I'd like to clarify. Partially, is this like, for example, the upfront payment and milestones? In terms of royalty, will you be posting in a different manner?

Ito [A]: We think that this is revenue that can be recorded on a recurring basis. Rather than upfront payment, we'd like to make it for JPY100 billion with the royalties. In terms of the upfront payments, it will be added on at the time when we conclude the agreement.

Sakai [Q]: Understood. With J-Brain Cargo, by 2030, there will be products coming out?

Ito [A]: Well, actually mid-2030, yes. There will be products, and we are expecting royalty revenue from that.

Sakai [Q]: Understood. For GH market, the novo nordisk situation is not clear to me, at least to me. 5 milligram supply was resumed, but it wasn't really selling well, I believe. Also, it was only for the particular adult market. As for Novo, not resuming their supply in a full-fledged manner. Once they are resumed, maybe once the patients have switched to your product and if they're stable on that product, they will, I'm sure they will continue under treatment with that product. Therefore, they won't switch back to Novo or Lilly. Therefore, with that, your sales will continue to grow, including capturing the share. Is that the preconditions that we can assume?

Shin Ashida [M]: Senior Vice President, Ashida, please.

Toru Ashida [A]: Well, thank you for your question. For your first question related to Novo, resuming the supply. This is the other company's issue. Therefore, I'd like you to actually ask Novo about their situation. There's already press release to health care professionals, and there are formulations which resumed its supply. After the supply resumed, not only Novo, but GROWJECT, as well as the new product that I presented,

and hospitals didn't have the trigger to adopt new products. In this supply issue, there are a lot of hospitals and GPs thanking GROWJECT.

Among them, with GROWJECT, for the first time, there are patients who use the motorized injector for the first time, as well as the co-medicals. Our injectors are the only motorized injectors in Japan. When patients use new injection, the device needs to be explained at each health care institution to co-medicals, and then that needs to be explained to patients. It was easier to actually explain, especially with GROWJECTOR Duo in patients using the injector for the first time and especially the pediatric patients using the app. They were pleased to find such a device.

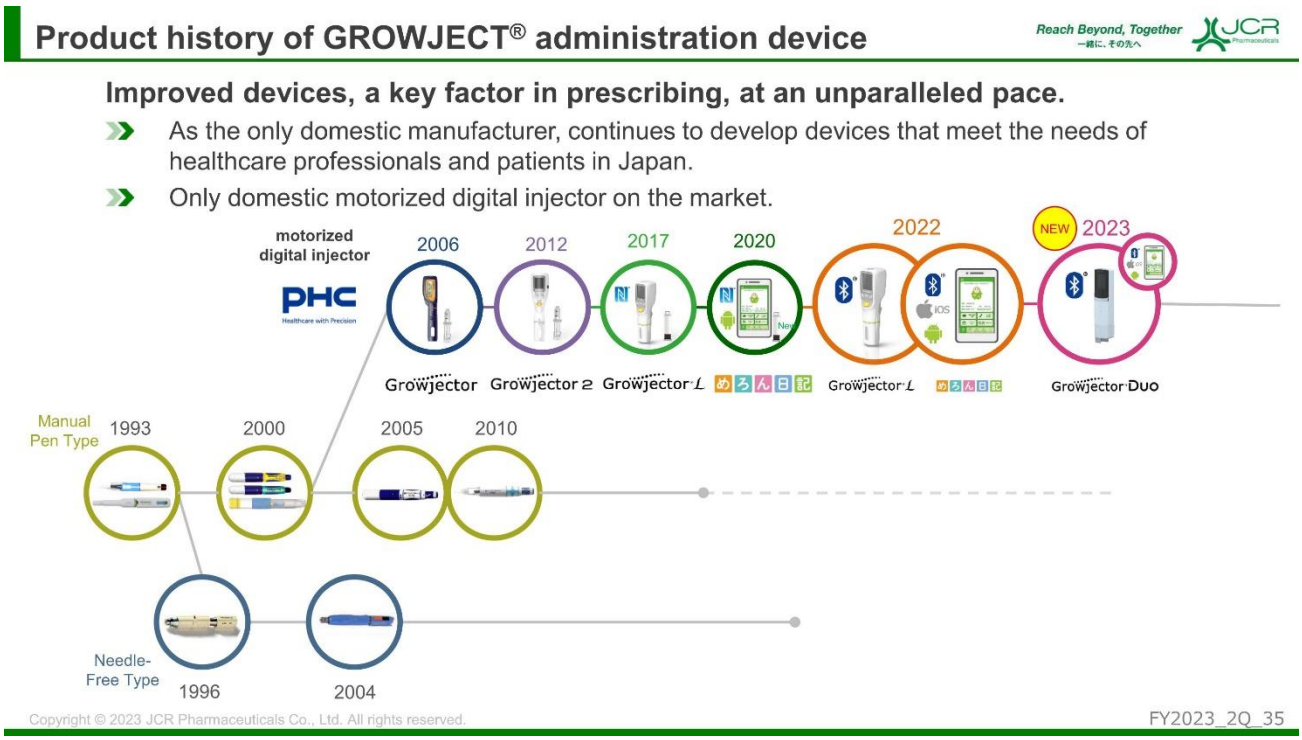
The doctors and health care professionals are also hearing great feedback from patients. Therefore, for the new patients that will be diagnosed, moving forward, GROWJECT will become an option to grow our share in the market, and that's our assumption.

Moderator [M]: Next question from Matsubara-san, please.

Matsubara [Q]: Matsubara from Nomura Securities. Thank you very much for your explanation. The first Novo, may restart the activities of what might come? That was the question. GROWJECTOR, how did the share change when the automation came about? When the Duo came about, how has the share changed? Would you please provide us with that information?

Shin Ashida [M]: Senior Vice President, Ashida, would you please respond?

Toru Ashida [A]: Thank you very much for your question. You are talking about the first electric device that we launched, right? When we launched that product, at that time, our target share, I'm sorry, I don't have the data right now. I cannot respond to this. I'm so sorry about this.



GROWJECTOR L, according to what I explained in my presentation, please take a look at this slide. At the top, you can see the first GROWJECTOR starting from 2006. We have the second generation, third generation, and Bluetooth with application and iOS, we added on the functions. Today, 2023, in September, we now have the

device Duo launched. When it comes to the concept, as I mentioned, this time, this year, because the supply issue came about, we did not expect that to happen.

With the GROWJECTOR Duo, we wanted to catch up on the HP, which has been a long-standing challenge for us because device explanation was known to be the hurdle barrier. Now, with this Duo, a much simpler function is available. With that device, we'll be able to expand our market in HP and also targeting to the older patients. With the Duo, our stance was to expand the HP market share.

I may not respond to your question. I'm so sorry, I'm not able to disclose the specific number as to how much market share was affected by these devices.

Matsubara [Q]: My second question, HIE. Standard of care and TEMCELL, there was no difference between the groups. Now, when it comes to the development into the future, whether you're going to stop the development or not. When is that decision going to be made? Maybe next year, would that be the case?

Shin Ashida [M]: Thank you very much. Sonoda is going to explain.

Sonoda [A]: Thank you very much for your question. TEMCELL, HIE, the indication expansion. As was mentioned, with that result from this, we are going to think about what we need to do. When and how, we have not decided that. Right now, we've been consulting with the investigators. Once it is decided at that timing, we are going to disclose them. Thank you very much.

Moderator [M]: Moving on to the next question, Tsuzuki-san, please.

Tsuzuki [Q]: This is Tsuzuki from Mizuho Securities. Materials, thank you very much for including a lot of information. My questions are about the weekly formulation of GH and development for the CNS area. The first one is a weekly formulation of GH. Currently, you have the results, but it's only the efficacy. Qualitatively, it's quite good. In terms of the differentiation points with other competitors, in terms of the safety, can you actually share some information on that?

Shin Ashida [M]: Well, thank you for your question. Then, Sonoda will take the question.

Sonoda [A]: Well, thank you for your question. The data that we showed today, this is the efficacy, the growth speed, but how is the safety compared to competitors, I believe, is the question. Regarding this, Phase I/II study data has been already published in paper. As a discussion, we have included the safety information as well as our thoughts. When GH is administered, IGF-1 actually working, but it shouldn't actually be excreted all the time. Therefore, we think it is preferable to mildly stimulate for the weekly formulation. From that perspective, we ourselves, we didn't have a head-to-head comparison study. This is only comparing the publication information. In terms of the safety profile, we believe that our product is superior to competitors' products.

Tsuzuki [Q]: Understood very well. The second question, in terms of developing into CNS area, on page seven. Regarding the J-Brain Cargo, with mAb or Fab or VHH, you have a lot of options. Currently, what is the direction in terms of your modality when applying J-Brain Cargo to CNS area? Is it like all parallel? Is one actually advancing further ahead?

Shin Ashida [M]: Sonoda will take this question as well.

Sonoda [A]: Thank you for your question. This is really difficult to answer. Internal data, we do have quite many. What I can say here is that the drug that we want to combine, what would be the best actually changes. Therefore, for any Fab or VHH is not the best. Therefore, individually, we have to look into what's best.

In terms of Fabs, we have different varieties. There's fine-tuning of, for example, one Fab. For example, before fine-tuning, VHH might be better. After that, Fab might be better. The optimization scheme is the most important, whether Fab or VHH. It's not what would be the most important, but there are so many pools that we have. In addition to that, we have the capability to fine-tune. I believe that is our advantage.

Tsuzuki [Q]: An additional question. On the VHH, is it also at the same level in terms of optimization as Fab?

Sonoda [A]: Yes, that is a correct understanding.

Moderator [M]: Our next question. Kawamura-san, please.

Kawamura [Q]: This is Kawamura from SBI Securities. For JR-141, enrollment situation and Last patient in timings. I think it's high time for me to confirm the schedule for those timings.

Shin Ashida [M]: JR-141. Maybe Matthias can respond.

Schmidt [A]*: Yes. We plan to conduct an interim analysis when 60% of the patients have been involved. We believe that enrollment is going to be achieved in the in H1 of FY2024. When it comes to the last patient in, for reasons that we don't want to disclose this information to our competitors, we rather want to refrain from answering this year. I kindly ask for your understanding on this.

Kawamura [Q]: Understood. Going to page six, the timeline and the revenue. Thank you very much for disclosing this. For the future, the ideas of the development, I would like to ask. Going down, you see the number of patients less and less. If you have 1,000 patients in global, double blinds are needed or Phase I/II would be enough or not. I'm aware about by looking at the number of patients, you are not able to make a clear comment. In terms of the timeline, how are you going to view this and interpret this table, which you could share?

Shin Ashida [M]: For this, Sonoda is going to respond.

Sonoda [A]: Thank you for a difficult question, to be honest. One by one, for each disease, development strategy is going to be different. As Kawamura-san mentioned, whether there is a drug available or not or maybe HSCT is going to be a workaround on that. That's another question. For each disease, what kind of clinical trial do we need to get? What kind of data do we need so that we'll be able to have an approval at the end of the day?

For rare diseases, the patient group at advocacy group and in the government regulatory negotiations are quite important because for this disease type, long-standing diseases, some diseases have patient advocacy groups, some do not. When it comes to the strategy, we have to be very specific. We have a strategy, like ideas, but we're not yet at the point of disclosing that information.

Kawamura [Q]: Thank you very much. As far as now, when the clinical trial is about to start, could you disclose any strategy?

Sonoda [A]: Yes, our ideas, to some extent, I can share that with you, but this is a pharmaceutical product, and the regulatory authority has control of approving it or not. As far as we are concerned, this is our schedule, and this is a clinical trial design that detailed programs, we can disclose when the time allows us to do so.

Moderator [M]: We are afraid that we are approaching the end of the briefing. We'd like to limit the questions to two more people. Next is Hashiguchi-san, please?

Hashiguchi [Q]: This is Hashiguchi from Daiwa Securities. The question that I wanted to ask was already asked similarly. I wanted to a refrain from asking because the response could not be made. Maybe from a different perspective, I'd like to ask a question even though it might be overlapping.

On page six, the table. From the beginning of the development to getting the approval, it looks like there are no variabilities between the compounds. My understanding is MPS II, there are variabilities in patients, and it takes more observations. On the other hand, MPS I, the assessment period could be shorter to show the efficacy because MPS I patient variability is not that large. MPS III, because CNS symptoms is critical and that assessment is a requirement. CNS symptoms, you cannot really see efficacy in a short period of time. Therefore, I expect a longer development in clinical settings.

Given those characteristics, how are you making the assumptions in terms of your plan? With the recent trend, we did see some shifts in the timeline presented before. The possibility of timeline being shifted moving forward, how did you formulate these? Is this like the mainstream scenario, progressive, or conservative? I'd like to ask those questions.

Shin Ashida [M]: Thank you for your question. For this as well, Sonoda will take the question.

Sonoda [A]: This is very similar to the previous question. It is really difficult for me to add information. Your understandings are correct. Especially the protocols by the competitors. How they are going to be and what kind of clinical results and what kind of clinical trials are done by the competitors. I'm sure that there are possibilities of changes in those aspects. Therefore, at this moment, this is very difficult to answer, what the assumptions are. In terms of the timeline and the angle that we have, as I mentioned, this is our rough estimate. Competitors' products as well as the future clinical results that we are getting, the timelines will shift. Depending on the results, the timeline may be shifted in quite possibility.

Moderator [M]: Then, the next question, Kubota-san, please.

Kubota [Q]: This is Kubota from Nikkei Biotechnology and Business. First question. Tsuzuki-san's earlier question, this has to do with the J-Brain Cargo. J-Brain Cargo can be used in a different way, depending on the situation. From the point of the quick response, what is actually the deciding point there? Effecter, in orange color on the slide, even though the enzyme may not be so effective directly, some other things can be attached or maybe a low molecule, something can be attached. Are you also thinking about the possibility of doing this?

Shin Ashida [M]: Sonoda will respond.

Sonoda [A]: The way we are using the J-Brain Cargo . As much as possible, not the PK (Pharmacokinetics), but PD (Pharmacodynamics). PD will be forecast for the selection. That's what we are thinking about. Why? Because even if you have different technology, only PK, it's very difficult to estimate PD. Depending on the disease, the effect expressed by PK is completely different. Of course, the modality is affect them. What kind of PK is appropriate at the subcellular level is also completely different depending on the drug. PD, the efficacy effectiveness is going to be focused.

Effecter, as you mentioned, is, in JR-141, an enzyme, in others, medicinal. So it could be the low molecules or maybe nucleic acid, and so on, depending on the situation. Already, at the J-Brain Cargo, a technology we have demonstrated has enzyme and the protein nucleic acids and lipid nanoparticle. We have already proven in our lab. When it comes to the effect, anything that can be fused or conjugated, anything is possible when it comes to this effect.

Kubota [Q]: The final question regarding that. In the third slide, biosimilar, the risk is low in the LSD. Nowadays, when we talk with pharmaceutical experts, the gene therapies and biosimilar may be difficult to produce. But

antibody, we have a biosimilar. Now, for the J-Brain Cargo, if you add enzyme to IgG type, you collectively generate it in CHO. However, VHH and scFv also the E. coli or the intestinal cells, the gene can be generated. The manufacturing way is very close to the antibody. there may be a possibility to have the biosimilar. Is that your thinking? Would that be possible?

Shin Ashida [A]: Well, when it comes to biosimilar, we said that biosimilar, the chance it is low. We say that because in the world, we have several hundred patients in this area. Having a biosimilar is quite difficult for any company. Very few do think such a way. In LSD, several thousand patients, several, maybe 100 or 200 patients, if you take a look at the segmentation of LSD. For the ultra-rare diseases, it is especially very difficult to generate any kind of a biosimilar by anyone.

Kubota [Q]: Rather than the modality, then that would really depend on the market, right?

Shin Ashida [A]; Yes. Even if they manufacture that, there might be a field in which the drug may not be effectively used. That's why the drugs may not be launched there.

Sonoda [A]: In terms of modalities Yes, your understanding is exactly, so the gene and cell therapies and the proteins, a number of them are there. In terms of the market size, we believe that the risk is quite low.

Moderator [M]: I'm afraid it is time. I would like to end the Q&A session. With this, we'd like to conclude JCR Pharmaceuticals briefing for FY2023 H1 financial results. Thank you very much for your participation.

[END]

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